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Pfizer Announces Positive Phase 3 Top-line Results for Once-Weekly Investigational Long-Acting Human Growth Hormone to Treat Children with Growth Hormone Deficiency

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– Results demonstrated improved treatment burden with once-weekly injection –

NEW YORK & MIAMI--(BUSINESS WIRE)-- Pfizer Inc. (NYSE: PFE) and OPKO Health Inc. (NASDAQ: OPK) announced today that C0311002, a Phase 3, randomized, multicenter, open-label, crossover study evaluating somatrogon dosed once-weekly in children 3 to <18 years of age with growth hormone deficiency (GHD), met its primary endpoint of improved treatment burden compared to GENOTROPIN® (somatropin) for injection administered once-daily.

This press release features multimedia. View the full release here: https://www.businesswire.com/news/home/20201008005030/en/

Top-line results from the study demonstrated that treatment with somatrogon once-weekly improved the mean overall Life Interference total score after 12 weeks of treatment (8.63) compared to treatment with somatropin administered once-daily (24.13). The point estimate of the treatment difference was -15.49 (-19.71, -11.27 (95% CI); $p<0.0001$) in favor of somatrogon at the nominal 0.05 level. In addition, key secondary endpoints showed an overall benefit in treatment experience with the somatrogon once-weekly dosing regimen compared to the somatropin once-daily dosing regimen.

“We’re encouraged by findings to date that demonstrate once-weekly somatrogon, if approved, has the potential to reduce lifestyle interference, support patient preference, and improve adherence as compared to a daily treatment,” said Brenda Cooperstone, MD, Chief Development Officer, Rare Disease, Pfizer Global Product Development. “Patients and their loved ones have endured the burden of daily growth hormone injections for nearly 40 years, and we are working to improve this current standard of care with a long-acting, weekly option for the growth hormone disorders community.”

No serious adverse events (SAEs) were reported during either of the treatment periods, and one participant discontinued use of somatrogon following a nonserious treatment-emergent adverse event (AE). The incidence of treatment-emergent AEs between the treatment arms were comparable and all AEs were mild to moderate in severity.

In 2014, Pfizer and OPKO entered into a worldwide agreement for the development and commercialization of somatrogon for the treatment of GHD. Under the agreement, OPKO is responsible for conducting the clinical

program and Pfizer is responsible for registering and commercializing the product. The companies will evaluate the potential for additional pediatric and adult indications, as appropriate.

About the Study

C0311002 is a Phase 3, randomized, multicenter, open-label, crossover study assessing subject perception of treatment burden with use of somatrogon administered once-weekly versus GENOTROPIN® administered once-daily in children 3 to <18 years of age with growth hormone deficiency (GHD). The primary objective of the crossover study, which included 87 randomized and treated subjects (43 randomized to Sequence 1 [somatropin followed by somatrogon] and 44 randomized to Sequence 2 [somatrogon followed by somatropin], was to evaluate the treatment burden of a somatrogon once-weekly injection schedule and a somatropin once-daily injection schedule, as assessed by the difference in mean overall Life Interference total scores after each 12-week treatment schedule experience.

About Somatrogon

Somatrogon is a new molecular entity that contains the natural sequence of growth hormone and one copy of the C-terminal peptide (CTP) from the beta chain of human chorionic hCG at the N-terminus and two copies at the C-terminus. The CTPs extend the half-life of the molecule. Somatrogon received Orphan Drug designation in the U.S. and the EU for the treatment of children and adults with growth hormone deficiency.

About Growth Hormone Deficiency

Growth hormone deficiency is a rare disease characterized by the inadequate secretion of growth hormone from the pituitary gland and affects one in approximately 4,000 to 10,000 people. In children, this disease can be caused by genetic mutations or acquired after birth. Because the patient's pituitary gland secretes inadequate levels of somatropin, the hormone that causes growth, his or her height may be affected and puberty may be delayed. Without treatment, he or she will have persistent growth attenuation, a very short height in adulthood, and may experience other health problems.

About GENOTROPIN® (somatropin)

GENOTROPIN (somatropin) is a man-made, prescription treatment option, approved in the United States for children who do not make enough growth hormone on their own, have the genetic condition called Prader-Willi syndrome (PWS), were born smaller than most other babies, have the genetic condition called Turner syndrome (TS) or have idiopathic short stature (ISS). GENOTROPIN is also approved to treat adults with growth hormone deficiency. GENOTROPIN is taken by injection just below the skin and is available in a wide range of devices to fit a range of individual dosing needs. GENOTROPIN is just like the natural growth hormone that our bodies make and has an established safety profile.

Important GENOTROPIN® Safety Information

- Growth hormone should not be used to increase height in children after the growth plates have closed.
- Growth hormone should not be used in patients with diabetes who have certain types of diabetic retinopathy (eye problems).
- Growth hormone should not be used in patients with cancer or who are being treated for cancer. Growth hormone deficiency can be caused by brain tumors. So, the presence of these brain tumors should be ruled out before treatment is started. Growth hormone should not be used if it is shown that a previous brain tumor has come back or is getting larger.

- Growth hormone should not be used in patients who are critically ill because of surgery, trauma, or respiratory failure.
- Growth hormone should not be used in children with Prader-Willi syndrome who are very overweight or have severe breathing problems.
- GENOTROPIN should not be used by patients who have had an allergy or bad reaction to somatropin or any of the other ingredients in GENOTROPIN.
- Some patients have developed diabetes mellitus while taking GENOTROPIN. Dosage of diabetes medicines may need to be adjusted during growth hormone treatment. Patients should be watched carefully if growth hormone is given along with glucocorticoid therapy and/or other drugs that are processed by the body in the same way.
- In childhood cancer survivors, treatment with growth hormone may increase the risk of a new tumor, particularly certain benign brain tumors. This risk may be higher in patients who were treated with cranial radiation. Also, patients and their doctors should check regularly for any skin changes.
- A small number of patients treated with growth hormone have had increased pressure in the brain. This can cause headaches and problems with vision. Treatment should be stopped and reassessed in these patients. Patients with Turner syndrome and Prader-Willi syndrome may be at higher risk of developing increased pressure in the brain.
- Thyroid function should be checked regularly during growth hormone therapy. Thyroid hormone replacement therapy should be started or adjusted if needed.
- Patients treated with growth hormone should be checked regularly if they are receiving standard hormone replacement therapy to treat a lack of more than one hormone.
- In children experiencing rapid growth, curvature of the spine may develop or worsen. This is also called scoliosis. Patients with scoliosis should be checked regularly to make sure their scoliosis does not get worse during their growth hormone therapy.
- In children experiencing rapid growth, limping or hip or knee pain may occur. If a child getting growth hormone therapy starts to limp or gets hip or knee pain, the child’s doctor should be notified and the child should be examined.
- Growth hormone should only be used during pregnancy if clearly needed. It should be used with caution in nursing mothers because it is not known whether growth hormone is passed into human milk.
- Use a different place on the body each day for growth hormone injections. This can help to prevent skin problems such as lumpiness or soreness.
- Some cases of pancreatitis (inflamed pancreas) have been reported rarely in children and adults receiving growth hormone. There is some evidence that there is a greater risk of this in children than in adults. Literature suggests that girls who have Turner syndrome may have a greater risk of pancreatitis than other children taking growth hormone. In any child who develops lasting, severe abdominal pain, pancreatitis should be considered.
- In studies of GENOTROPIN in children with GHD, side effects included injection site reactions, such as pain, redness/swelling, inflammation, bleeding, scarring, lumps, or rash. Other side effects were fat loss, headache, blood in the urine, low thyroid activity, and mildly increased blood sugar.
- In studies of GENOTROPIN in children born SGA, side effects included temporarily elevated blood sugar, increased pressure in the brain, early puberty, abnormal jaw growth, injection site reactions, growth of moles, and worsening of scoliosis (curvature of the spine).
- Deaths have been reported with the use of growth hormone in children with Prader-Willi syndrome. These children were extremely overweight, had breathing problems, and/or lung infection. All patients with Prader-Willi syndrome should be examined for these problems. They should also establish healthy weight control.
- In studies of GENOTROPIN in children with PWS, side effects included fluid retention, aggressiveness, joint and muscle pain, hair loss, headache, and increased pressure in the brain.

- Turner syndrome patients taking growth hormone therapy may be more likely to get ear infections. This is also called otitis media.
- In studies of GENOTROPIN in children with Turner syndrome, side effects included flu, throat, ear, or sinus infection, runny nose, joint pain, and urinary tract infection.
- In studies of GENOTROPIN in children with ISS, side effects included respiratory illnesses, flu, throat infection, inflammation of the nose and throat, stomach pain, headaches, increased appetite, fever, fracture, mood changes, and joint pain.
- Women who are taking estrogen by mouth may take GENOTROPIN. They may need a larger dose of growth hormone.
- GENOTROPIN may be taken by the elderly. Elderly patients may be more likely to have side effects with growth hormone therapy.
- In studies of GENOTROPIN in adults with GHD, side effects included fluid retention, joint or muscle pain, stiffness, and changes in sensation. Usually these side effects did not last long and depended on the dose of GENOTROPIN being taken.
- GENOTROPIN cartridges contain m-Cresol and should not be used by patients allergic to it.
- A health care provider will help you with the first injection. He or she will also train you on how to inject GENOTROPIN.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088.

For the full Prescribing Information for GENOTROPIN, please visit http://labeling.pfizer.com/ShowLabeling.aspx?id=577.

GENOTROPIN Indications and Usage in the U.S.

GENOTROPIN is a prescription product for the treatment of growth failure in children:

- Who do not make enough growth hormone on their own. This condition is called growth hormone deficiency (GHD).
- With a genetic condition called Prader-Willi syndrome (PWS). Growth hormone is not right for all children with PWS. Check with your doctor.
- Who were born smaller than most other babies born after the same number of weeks of pregnancy. Some of these babies may not show catch-up growth by age 2 years. This condition is called small for gestational age (SGA).
- With a genetic condition called Turner syndrome (TS).
- With idiopathic short stature (ISS), which means that they are shorter than 98.8% of other children of the same age and sex; they are growing at a rate that is not likely to allow them to reach normal adult height, and their growth plates have not closed. Other causes of short height should be ruled out. ISS has no known cause.

GENOTROPIN is a prescription product for the replacement of growth hormone in adults with growth hormone deficiency (GHD) that started either in childhood or as an adult. Your doctor should do tests to be sure you have GHD, as appropriate.

About OPKO Health, Inc.

OPKO is a multinational biopharmaceutical and diagnostics company that seeks to establish industry-leading positions in large, rapidly growing markets by leveraging its discovery, development, and commercialization expertise and novel and proprietary technologies. For more information, visit http://www.OPKO.com.

About Pfizer Rare Disease

Rare disease includes some of the most serious of all illnesses and impacts millions of patients worldwide, representing an opportunity to apply our knowledge and expertise to help make a significant impact on addressing unmet medical needs. The Pfizer focus on rare disease builds on more than two decades of experience, a dedicated research unit focusing on rare disease, and a global portfolio of multiple medicines within a number of disease areas of focus, including rare hematologic, neurologic, cardiac and inherited metabolic disorders.

Pfizer Rare Disease combines pioneering science and deep understanding of how diseases work with insights from innovative strategic collaborations with academic researchers, patients, and other companies to deliver transformative treatments and solutions. We innovate every day leveraging our global footprint to accelerate the development and delivery of groundbreaking medicines and the hope of cures.

Click here to learn more about our Rare Disease portfolio and how we empower patients, engage communities in our clinical development programs, and support programs that heighten disease awareness.

Pfizer Inc.: Breakthroughs that change patients' lives

At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products, including innovative medicines and vaccines. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world's premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For more than 150 years, we have worked to make a difference for all who rely on us. We routinely post information that may be important to investors on our website at www.pfizer.com. In addition, to learn more, please visit us on www.pfizer.com and follow us on Twitter at @Pfizer and @Pfizer_News, LinkedIn, YouTube and like us on Facebook at Facebook.com/Pfizer.

DISCLOSURE NOTICE:

The information contained in this release is as of October 6, 2020. Pfizer and OPKO assume no obligation to update forward-looking statements contained in this release as the result of new information or future events or developments.

This release contains forward-looking information about an investigational growth hormone deficiency therapy, somatrogon, including its potential benefits, that involves substantial risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statements. Risks and uncertainties include, among other things, the uncertainties inherent in research and development, including the ability to meet anticipated clinical endpoints, commencement and/or completion dates for our clinical trials, regulatory submission dates, regulatory approval dates and/or launch dates, as well as the possibility of unfavorable new clinical data and further analyses of existing clinical data; the risk that clinical trial data are subject to differing interpretations and assessments by regulatory authorities; whether regulatory authorities will be satisfied with the design of and results from our clinical studies; whether and when drug applications for any potential indications for somatrogon may be filed in any jurisdictions; whether and when regulatory authorities in any jurisdictions may approve any such applications, which will depend on myriad factors, including making a determination as to whether the product's benefits outweigh its known risks and determination of the product's efficacy and, if approved, whether somatrogon will be commercially successful; decisions by regulatory

authorities impacting labeling, manufacturing processes, safety and/or other matters that could affect the availability or commercial potential of somatrogon; uncertainties regarding the impact of COVID-19 on Pfizer's or OPKO's business, operations and financial results; and competitive developments.

A further description of risks and uncertainties can be found in Pfizer's and OPKO's respective Annual Reports on Form 10-K for the fiscal year ended December 31, 2019 and in their respective subsequent reports on Form 10-Q, including in the sections thereof captioned "Risk Factors", "Forward-Looking Information and Factors That May Affect Future Results" and "Cautionary Statement Regarding Forward-Looking Statements", as well as in their respective subsequent reports on Form 8-K, all of which are filed with the U.S. Securities and Exchange Commission and available at www.sec.gov and, as applicable, www.pfizer.com and www.OPKO.com.

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